

IMI2

18th Call for proposals

**Annex II to the Decision of the IMI2 JU Governing Board No. IMI2-GB-DEC-2019-12
adopted on 21 June 2019**

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Introduction

The Innovative Medicines Initiative is a jointly funded partnership between the European Union, represented by the European Commission, and the European Federation of Pharmaceutical Industries and Associations (EFPIA).

The Innovative Medicines Initiative 2 Joint Undertaking (IMI2 JU) has been created¹ following the principles below:

Research related to the future of medicine should be undertaken in areas where societal, public health and biomedical industry competitiveness goals are aligned and require the pooling of resources and greater collaboration between the public and private sectors, with the involvement of Small and Medium-sized Enterprises (SMEs).

The scope of the initiative should be expanded to all areas of life science research and innovation.

The areas should be of public health interest, as identified by the World Health Organisation (WHO) report on priority medicines for Europe and the World².

The IMI2 JU objectives are usually implemented through Research and Innovation Actions (RIAs), and Coordination and Support Actions (CSAs) where public and private partners collaborate, joining their expertise, knowledge and resources.

The initiative should therefore seek to involve a broader range of partners, including mid-sized companies³, from different sectors e.g. biomedical imaging, medical information technology, diagnostic and/or animal health industries. Involving the wider community in this way should help to advance the development of new approaches and technologies for the prevention, diagnosis and treatment of diseases with high impact on public health.

The IMI2 Strategic Research Agenda (SRA)⁴ is the main reference for the implementation of research priorities for IMI2 JU. The scientific priorities for 2019 for IMI2 JU have been prepared based on the SRA.

Applicant consortia are invited to submit a proposal for each of the topics that are relevant for them. These proposals should address all aspects of the topic to which the applicant consortia are applying. The size and composition of each consortium should be adapted so as to respond to the scientific goals and the expected key deliverables.

Applicant consortia, during all stages of the evaluation process, must consider the nature and dimension of the IMI2 JU programme as a public-private collaboration.

While preparing their proposals, applicant consortia should ensure that the needs of patients are adequately addressed and, where appropriate, patient involvement is encouraged. Applicants should ensure that gender dimensions are also considered. Synergies and complementarities with other national and international projects and initiatives should be explored in order to avoid duplication of efforts and to create collaboration at a global level to maximise European added value in health research. Where appropriate, the involvement of regulators is also strongly encouraged.

¹ Council Regulation (EU) No 557/2014 of 6 May 2014 establishing the Innovative Medicines Initiative 2 Joint Undertaking (IMI2 JU), OJ L 169, 7.6.2014, p. 54–76.

² http://www.who.int/medicines/areas/priority_medicines/en/

³ Under IMI2 JU, mid-sized companies having an annual turnover of EUR 500 million or less not being affiliated entities of companies with an annual turnover of more than 500 million; the definition of 'affiliated entities' within the meaning of Article 2(1)(2) of Regulation (EU) No 1290/2013 applies mutatis mutandis. Where established in an EU Member State or an associated country, are eligible for funding.

⁴ http://www.imi.europa.eu/sites/default/files/uploads/documents/About-IMI/research-agenda/IMI2_SRA_March2014.pdf

Applicant consortia shall ensure that where relevant their proposals are in compliance with the General Data Protection Regulation (EU) 2016/679⁵ and Clinical Trial Regulation (EU) 536/2014⁶ (and/or Directive 2001/20/EC⁷) and any relevant legislation⁸.

Before submitting a proposal, applicant consortia should familiarise themselves with all Call documents such as the IMI2 JU Manual for submission, evaluation and grant award⁹, and the IMI2 evaluation criteria. Applicants should refer to the specific templates and evaluation procedures associated with the topic type Research and Innovation Actions (RIA).

⁵ Regulation (EU) 2016/679 of the European Parliament and of the Council of 27 April 2016 on the protection of natural persons with regard to the processing of personal data and on the free movement of such data, and repealing Directive 95/46/EC (General Data Protection Regulation), OJ L 119, 4.5.2016, p. 1–88.

⁶ Regulation (EU) No 536/2014 of the European Parliament and of the Council of 16 April 2014 on clinical trials on medicinal products for human use, and repealing Directive 2001/20/EC, OJ L 158, 27.5.2014, p. 1-76.

⁷ Directive 2001/20/EC of the European Parliament and of the Council of 4 April 2001 on the approximation of the laws, regulations and administrative provisions of the Member States relating to the implementation of good clinical practice in the conduct of clinical trials on medicinal products for human use (the "Clinical Trials Directive"), OJ L 121, 1.5.2001, p. 34.

⁸ Directive 95/46/EC on the protection of individuals with regard to the processing of personal data and the free movement of such data and implementing national laws, OJ L 281, 23.11.1995, p. 31–50.

⁹ https://www.imi.europa.eu/sites/default/files/uploads/documents/apply-for-funding/call-documents/imi2/IMI2_ManualForSubmission_v1.7_November2018.pdf

Topic 2: Health Outcomes Observatories – empower patients with tools to measure their outcomes in a standardised manner creating transparency of health outcomes

Topic details

Topic code	IMI2-2019-18-02
Action type	Research and Innovation Action (RIA)
Submission and evaluation process	2 stages

Specific challenges to be addressed

Patient outcomes and their experience of healthcare, and thus their overall care, could be improved through systematic capture of the patient voice and perspective.

There is general agreement on the need for increased patient centricity in healthcare provision. Current conceptualisations and measures of disease and clinically relevant disease outcomes have generally been developed from the perspective of the clinician and often fail to completely capture the totality of the disability, the symptoms of the disease and the impact on a patient's health-related quality of life (HRQOL) and a patient's experience of their healthcare from the patient's perspective. Important patient-to-patient variations in disease presentation and symptomology may also be lost in the effort to develop a generalisable framework for the disease.

It is important to complement existing clinical outcome measurements with patient-generated measures of disease and HRQOL to ensure that the patient perspective of disease and the impact of healthcare interventions are more completely captured and that disease heterogeneity is better understood. Patient-reported outcomes (PROs) are significant indicators for quality of life and quality of treatment. Their medical and psychological impact has been described for a broad range of diseases. A fine balance must be struck between maintaining authenticity and faithfully capturing the voice of the patient and making the data collected interpretable and generalisable.

In order to achieve this, it is essential to provide patients with tools that have the ability to better capture the entirety of the impact of a disease and treatments (e.g. signs, symptoms, tolerability), allowing them to document their disease more completely and in a structured manner. To be effective, these tools should be built on the basis of accepted standards, developed in partnership with all relevant stakeholders and accepted and integrated into the existing healthcare ecosystem.

A reward system that truly focuses on value requires measurement and transparency of patient outcomes.

Healthcare systems that have the goal of rewarding innovators and service providers on the basis of the value they create for patients need to collect transparent and reliable data on outcomes. Disease registries have already been established in a wide range of diseases. However, these registries tend to measure a non-standardised set of outcomes, are rarely interoperable, focus on clinical measurements, and have varying terms and conditions for access to the data captured. As a result, they often fall short of providing sufficient transparency of patient outcomes in specific diseases to inform scientific and policy decisions.

At the level of the individual patient, the data generated, once structured and subjected to a degree of standardisation, will enable patients to have more productive interactions with their healthcare provider. At the level of the healthcare system, this data will allow a systematic measurement of health outcomes and the possibility to set up a reward system based on value – which can be defined as the level of health outcomes achieved for a given cost.

There is a lack of models for capturing and managing patient-reported health data in an ethical and sustainable way.

Structured health data is invaluable for all stakeholders, from the individual patient, healthcare professionals (HCPs), the life science industry, and policy makers to the patient advocacy groups. There have been a few successful examples of approaches to integrate patient-reported health data into clinical care. In an era of greater focus on the patient, it is thus critical for a society that patient-reported health and experience data is captured and managed in an ethical manner ensuring broad and appropriate access while safeguarding patients' privacy and building high levels of trust.

Need and opportunity for public-private collaborative research

Despite rapid advances in medical science and a revolution in health technology, the lack of standardisation and integration of data remains an obstacle to fully realising the promised benefits of the digital revolution¹¹. Measurement methodologies and outcome standards need to be endorsed by those both generating the data and those using the data, and be part of the broader healthcare ecosystem in order to be trusted and accepted. The complexity of the challenges is such that it requires action that is collective, innovative and nurtured in an environment where sensitive information can be shared securely.

- patient associations need to engage actively to develop tools and approaches, and to build trust and patient engagement.;
- regulatory authorities need to be part of the dialogue regarding novel endpoints, data requirements, and acceptability of evidence from patient-generated data;
- privacy and legal experts need to set up the appropriate governance models, consent forms and access terms in order to allow data sharing, ensure trust and, therefore, support sustainability;
- life sciences companies are critical, not only for bringing in expertise, commitment to long-term research, innovation and evidence generation in the disease areas, but also for providing funding and ensuring that the model can be made sustainable over the long term;
- small and medium-sized enterprises (SMEs) and other innovators such as digital companies need to be involved to develop the appropriate tools and technologies;
- public sector experts including medical experts, ethicists, social scientists, biostatisticians and researchers are required to identify or develop the appropriate measurements and the right methodologies for capturing and analysing the data;
- data custodians and data management experts are also essential.

Scope

The goals of this topic are as follows:

1. identify appropriate standards for capturing the patient perspective when measuring health outcomes and patients' experience of healthcare, and obtain support for these standards among relevant stakeholders. Where appropriate the partners will give preference to standards already being developed (e.g. International Consortium for Health Outcomes Measurement - ICHOM) and will follow the Observational Medical Outcomes Partnership Common Data Model (OMOP CDM) developed through Observational Health Data Sciences and Informatics (OHDSI);
2. implement appropriate technology solutions (including adopting existing technology where appropriate) that would allow individual patients to record and measure their outcomes according to these standards and use the information for a more structured dialogue with their HCPs. The technical solution developed will

¹¹ As acknowledged by the OECD in their paper: Fujisawa, R. and N. Klazinga (2017), "Measuring patient experiences (PREMS): Progress made by the OECD and its member countries between 2006 and 2016", OECD Health Working Papers, No. 102, OECD Publishing, Paris, <https://doi.org/10.1787/893a07d2-en>

make extensive use of smartphones and/or other commercially available wearable devices to collect both patient outcome measures and objective measures of patient function;

3. establish the appropriate platform to collect, process and manage data in the best interest of patients, patient organisations, health authorities, healthcare professionals, the research community and health care payers, and in compliance with General Data Protection Regulation (GDPR) and other relevant rules and regulations;
4. create a sustainable, socially acceptable and ethical model for the continuous collection of data and an appropriate model for providing access to the identifiable or anonymised or aggregated data to researchers with a legitimate interest in analysing them.

These goals can be achieved through the creation of a consortium whose mission will be to establish health outcomes observatories in three selected disease areas, collecting health data in (at least) three different European countries¹² for each disease area. It would be desirable for the three countries selected to reflect variability across Europe in order to provide experience and guidance for scaling the initiative more effectively to other countries in the future.

The observatories should be designed according to the following principles:

- full integration within the respective countries' healthcare systems;
- consistency in design across observatories to allow for comparability of patient outcomes across countries;
- a sustainable model for the observatories;
- robust patient consent and engagement;
- standardisation and interoperability across countries.

The disease areas selected are:

- diabetes type 1 and type 2;
- inflammatory bowel disease (IBD);
- cancer (side effects of chemotherapy and immuno-oncology).

Criteria considered for this selection were: (a) their prevalence in the European population; (b) their chronic and progressive nature; (c) their significant impact on patients' quality of life; (d) their compatibility with patients' digital literacy; (e) the patients have sufficient autonomy and motivation to become engaged in self-management of their disease; and (f) the investment in novel medicines and disease management tools for these diseases by EFPIA members and IMI Associated Partners. The disease areas will focus on adult patients.

Expected key deliverables

The overall aim is the creation and operation of observatories in (at least) the three disease areas identified collecting health data in (at least) three different European countries. The deliverables from the project funded under this topic would all be made public and a key objective is to set up the observatories on a sustainable basis.

To achieve this, the applicants will have to focus on the following deliverables:

- an appropriate, societally accepted, governance and sustainability model for the observatories in three different European countries that allows inclusion in the respective national health ecosystem, and develops revenue streams to fund the continued operation of the observatories beyond the life of the initial project term;

¹² European Union and H2020 Associated countries

- all legal and ethical analysis required to ensure appropriate consents for data collection, data management and access terms and conditions;
- the legal set up and operation of the observatories, sustainable beyond the life of the initial project term;
- the design and set-up of the appropriate infrastructure leveraging where possible existing technological solutions that would allow the collection of patient-generated data using an accepted common data model (e.g. OMOP CDM);
- the design of a methodology for identifying the appropriate measurements of outcomes for respective diseases taking into consideration the need to also ensure broad stakeholder acceptability and comparability of these measurements;
- the identification of the appropriate measurements of outcomes for the focus diseases of this project and the creation of an adequate digital tool leveraging as much as possible existing solutions;
- the launch of the respective digital tools;
- the publication of annual reports after the third year comparing health outcomes in the three European countries and identifying lessons learned and opportunities for improvement.

For the three specific disease areas, the work will focus on the following deliverables:

- identification and validation of key outcome measures to inform health economic evaluations in the disease area;
- analysis of patient outcome data in combination with electronic health records by means of advanced methodologies for patient stratification to determine ideal levels of care;
- a digital decision-making system based on the stratification above to allow personalised treatment.

Expected impact

Applicants should describe how the outputs of the project will contribute to the following impacts and include baseline, targets and metrics to measure impact:

- enable individual patients to:
 - receive close to real-time information on their disease status;
 - hold more informed discussions with healthcare professionals about their health status and options;
 - better understand how their status compares with other patients with a similar condition;
 - share their data and help the broader patient community.
- allow healthcare professionals to:
 - track the evolution of their patients;
 - enable a different outcome-based conversation;
 - better inform and enhance clinical decisions based on the patient perspective.
- allow patient organisations to:
 - assess the status and dynamics of their patient population;
 - increase engagement with other healthcare stakeholders in evidence-based advocacy;
 - further contribute to improving the healthcare system.
- allow health authorities and healthcare providers to:
 - improve the quality of care through better and more transparent evidence of patient measures and outcomes;
 - drive research agendas and investments in the right areas;

- ensure the sustainability of healthcare systems in finding ways to improve the allocation of resources.
- allow pharmaceutical companies and other innovative companies to use data to:
 - enable ethical utilisation of the observatory data as legally appropriate;
 - generate insights that can be used to support the design and direction of the development of new treatments;
 - generate robust evidence that can be used in submissions to regulators, health technology assessment (HTA) agencies and other decision makers.

It is also expected that the pool of harmonised data that will be generated can be shared with other institutions and consortia (see section 'Potential synergies with existing Consortia'). Standardised data across geographies can eventually enable comparison of outcomes among different healthcare systems.

Finally, applicants should indicate how their proposal will impact the competitiveness and industrial leadership of Europe by, for example, engaging suitable SMEs.

Potential synergies with existing Consortia

Applicants should take into consideration, while preparing their short proposal, relevant national, European (both research projects as well as research infrastructure initiatives), and non-European initiatives. Synergies and complementarities should be considered in order to incorporate past achievements, available data and lessons learnt where possible, thus avoiding unnecessary overlap and duplication of efforts and funding.

Collaboration agreements

There is the potential for important synergies between the consortium selected under this topic and the one selected under IMI2 JU Call 18 topic 3 (*Improving patient access, understanding and adherence to healthcare information: an integrated digital health information project*). In particular, on the one hand, for instance it could be possible for the consortium selected under topic 2 to leverage the observatory platform in order to obtain access to and analyse relevant electronic health record (EHR) data, in compliance with applicable regulation, gathered under topic 3. On the other hand, the consortium selected under topic 3 could become an additional important use-case for the observatories and improve their usefulness. Additionally, the perspectives brought by the consortium selected under topic 3 can contribute to development of the governance and operational model of the observatories, under topic 2. It could also help future-proof them as a neutral guardian of patients' health data which could then be made available in the future with the appropriate safeguards for applications, such as those envisaged under topic 3.

To explore these potential synergies between actions funded under these two topics, the selected consortia are expected to cooperate in common boards/structures and provide access to their results for specific activities when relevant. Therefore the grants awarded under IMI2 JU Call 18 topics 2 and 3 will be complementary grants. The respective options under Article 2, Article 31.6 and Article 41.4 of the IMI2 JU Model Grant Agreement¹³ will apply. Accordingly, the relevant consortia will conclude collaboration agreement(s) to ensure the exchange of relevant information, exploration of synergies, collaboration where appropriate.

Other potential synergies

The project funded under this topic will build on applicable methodologies and principles established in particular (but not limited to):

¹³ See: https://www.imi.europa.eu/sites/default/files/uploads/documents/reference-documents/h2020-mga-imi_en_v5.pdf

- Projects from the IMI2 Big Data for Better Outcomes (BD4BO) programme such as:
 - [EHDEN](#) – for infrastructure and principles of data standardisation;
 - [BD4BO](#) disease-specific projects – for their principles of establishing the usefulness of PROs and real world evidence (RWE) in decision making and establishing the value of interventions;
 - [DO-IT](#) – for its informed consent principles and recommendations amongst others Patient
- engagement projects such as [EUPATI](#) and [PARADIGM](#);
- OMOP CDM (OHDSI) can provide a common model to encode data as well as important analytical tools.
- Projects suggesting novel treatment options and establishing patient survey mechanisms (e.g. [BIOCYCLE](#)).

Industry consortium

The industry consortium is composed of the following EFPIA companies:

- Takeda (lead)
- AbbVie
- Eli Lilly
- Hoffmann-La Roche Ltd
- Medtronic
- Pfizer
- Sanofi
- Novartis

In addition, the industry consortium includes the following IMI2 JU Associated Partners:

- Juvenile Diabetes Research Foundation (JDRF)
- Trial Nation

The industry consortium will contribute to the 'horizontal phase' of the project by providing the following expertise:

- medical knowledge for the disease areas;
- regulatory expertise;
- health outcomes and RWE expertise;
- legal expertise;
- financial and business planning expertise;
- digital technologies expertise;
- expertise in public-private partnerships related to clinical research in the health care ecosystem.

This expertise will be provided for the following tasks to be executed in collaboration with the public consortium:

- identification/ design of the underlying requirements (medical, legal, regulatory, etc.);
- business plan including governance model, structure, and sustainability;
- interactions with regulators and health care authorities for the acceptability of the PROs and of the observatories;
- selection of the digital technologies to measure PROs;

- development of methods to analyse the PROs.

Moreover, the industry consortium will contribute to the disease-specific 'vertical phase' by providing medical and regulatory experts for the disease areas, as well as expertise in digital technologies, health outcomes and RWE.

Indicative duration of the action

The indicative duration of this action is 60 months.

Future project expansion

Potential applicants must be aware that the Innovative Medicines Initiative 2 (IMI2) Joint Undertaking may, if exceptionally needed, publish at a later stage another Call for proposals restricted to the consortium already selected under this topic in order to enhance and progress the results and achievements by extending action duration and funding. The consortium will be entitled to open to other beneficiaries as it sees fit.

In the context of this topic, a restricted Call may be launched as part of a future IMI2 JU Annual Work Plan to expand the work to include additional data sources, therapeutic areas and/or health economic analysis, leveraging the success achieved. This would help to maximise the long-term impact of the project and to engender continued future successes in making outcomes and value concepts and their application in healthcare and clinics being more fruitful and efficient.

Indicative Budget

The indicative in-kind and financial contribution from EFPIA partners and IMI2 JU Associated Partners is EUR 11 435 000.

This contribution comprises an indicative EFPIA in-kind contribution of EUR 10 385 000, of which EUR 900 000 financial contributions, and an indicative IMI2 Associated Partners in-kind contribution of EUR 1 050 000, of which 882 000 financial contributions.

The allocation of the financial contribution from EFPIA partners and Associated Partners to the beneficiaries receiving JU funding will be decided by the full consortium at stage 2 when preparing the full proposal.

Due to the global nature of the participating industry partners and IMI2 JU Associated Partner, it is anticipated that some elements of the contributions will be non-EU/H2020 Associated Countries in-kind contributions.

The financial contribution from IMI2 JU is a maximum of EUR 10 478 000.

Applicant consortium

The applicant consortium will be selected on the basis of the submitted short proposals.

The applicant consortium is expected to address all the objectives and to make key contributions to the defined deliverables in synergy with the industry consortium which will join the selected applicant consortium in preparation of the full proposal for stage 2.

This may require the applicant consortium to mobilise, as appropriate, the following expertise:

- solid experience in measuring health outcomes, creating appropriate methodologies that allow the capture of patient insights and integrating these methodologies appropriately to gain broad acceptance;
- access to existing real-world data and technology to query the data, together with experience in creating and operating patient registries that capture patient's input and preferences;

- demonstrated ability to build strong relationships with the health authorities and patient organisations of at least three different European countries where there is desire and willingness to co-create these observatories together with the industry;
- strong legal skills including GDPR / data governance aspects but also in broader healthcare law;
- digital architecture and technical skills, including data linkage skills, to set up and/or adapt and operate the appropriate infrastructure in full compliance with GDPR and cybersecurity requirements;
- technical capabilities to create the right digital solutions that will allow individual patients to monitor their outcomes in accordance with the agreed standards;
- expertise in data mining, machine learning, computational biology and modelling expertise and resources;
- biostatisticians and epidemiologists to combine and analyse the data and publish regularly on the outcomes;
- medical expertise across the disease areas;
- social scientists to ensure a robust and socially acceptable model for the collection of data;
- expertise in planning, developing and drafting communications to a range of audiences (including, but not limited to, medical, patient, academic and policy maker audiences);
- strong project management expertise.

Very importantly, the applicant consortium should include among their participants, either as members of the consortium or demonstrated willingness to contribute as experts:

- patient advocacy groups in the respective disease areas and the respective countries to ensure that the patient voice is appropriately heard, captured and interpreted;
- national bodies, such as regulatory agencies and/or HTA agencies and/or health authorities in the respective countries/regions to ensure that the observatories will become part of the national/regional healthcare ecosystems.

Data management

In their short proposal, applicants should give due visibility to 'data management'. At stage 2, applicants should include a draft data management plan (DMP) in the full proposal, outlining how research data will be handled and made available during the project and after it is completed.

Dissemination, exploitation and communication activities

In their short proposal, applicants should give due visibility to the dissemination, exploitation and communication of the project's results. At stage 2, in their full proposal, applicants should further develop these activities.

Partnership with the industry consortium

In their short proposal, applicants should outline a strategy to create a successful partnership with the industry consortium.

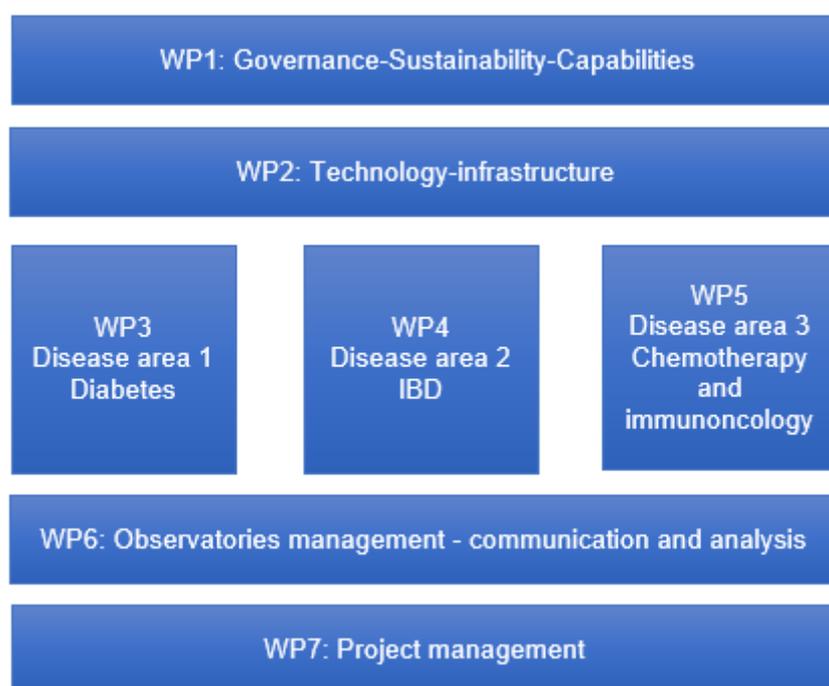
Suggested architecture of the full proposal

The applicant consortium should submit a short proposal which includes their suggestions for creating a full proposal architecture, taking into consideration the industry participation including their contributions and expertise provided below.

In the spirit of the partnership, and to reflect how IMI2 JU Call topics are built on identified scientific priorities agreed together with EFPIA beneficiaries/large industrial beneficiaries, these beneficiaries should significantly contribute to the programme and project leadership as well as project financial management. The final architecture of the full proposal will be defined by the participants in compliance with the IMI2 JU rules and with a view to the

achievement of the project objectives. The allocation of a leading role within the consortium will be discussed in the course of the drafting of the full proposal to be submitted at stage 2. To facilitate the formation of the final consortium, until the roles are formally appointed through the consortium agreement, the proposed project leader from among EFPIA beneficiaries/large industrial beneficiaries shall facilitate an efficient negotiation of project content and required agreements. All beneficiaries are encouraged to discuss the project architecture and governance and the weighting of responsibilities and priorities therein.

The architecture outlined below for the full proposal is a suggestion. The architecture of the full proposal should be designed to fulfil the objectives and key deliverables within the scope of this proposal.



Work package 1: Governance - Sustainability - Capabilities

- design of the specific governance principles and structures including legal structures, funding and operating model in the given countries in a way to ensure long-term sustainability. This should include the governance and operating procedures for the creation and maintenance of the observatories, including their relationship with patient organisations, health authorities both at regional/national and above country level and commercial entities. Important elements for the design of the appropriate governance model would be:
 - the long-term sustainability of the model;
 - the possibility to scale it to further disease areas;
 - the interoperability of the data collected with health data derived from EHRs, registries, academic researchers, etc.;
 - the development of a robust consenting process in compliance with the GDPR and other relevant legal and regulatory requirements;
 - the creation of an ethics council to watch over the observatories to build strong trust levels among patients and society.
- methodology for identifying the appropriate measurement standards ensuring they reflect patients' priorities and validating them. In order for any measurement/reporting tool to be truly useful to patients, it should

offer them the opportunity to improve their communication with their HCP and/or the healthcare system more broadly. It is therefore an important part of the mission of the observatories to choose standards that reflect patients' priorities but also integrate these standards with the broader stakeholders in order to gain broader acceptability;

- identify the capabilities and capacity required for the collection, analysis and dissemination of health data in the observatories, including the required capabilities for data analysis and administration, and staff the observatories appropriately.

Work package 2 – Technology – Infrastructure

Identify the appropriate technology that will allow the capture of relevant information from patients and enable real-time information sharing with patients. Set up or adapt the appropriate technology, including tools and a platform, that would allow the collection and management of patient-generated data taking into consideration the possible scalability of the project as well as the interoperability of this data with health data derived from other sources (EHR, registries etc.).

Work packages 3 – 5

These work packages will focus on each disease area, aiming to enhance the value of treatment and care for patients through the collection of patient-generated data, the analysis of best care practices as well as the development and validation of digital e-health tools and technologies. The ultimate aim is to increase the wellbeing of patients through improvements in patient care that have been developed with greater insights from patients generated by the observatories.

Specific common objectives are:

- identify the appropriate measurement standard for the respective disease/outcome and ensure validation by the stakeholder community;
- create the methodology to answer the specific research questions identified by the consortium as the most pertinent to the respective disease;
- provide input to the design of technologies in WP2.

Work package 3 – Diabetes types 1 and 2

- to focus on the analysis and validation of key outcomes measures and assess their usefulness for diabetes care and contribution to health economic aspects of the healthcare system;
- to use state of the art analytical techniques to demonstrate ideal levels of care based on the validated outcomes data together with other data types such as EHR and patient-generated data;
- to stratify people with diabetes according to outcomes to improve the understanding of diabetic endotypes;
- to develop a digital decision-making system which can be used by healthcare professionals in clinical practice for more personalised treatment of people with type 1 and type 2 diabetes.

The following sub-work packages are proposed to achieve WP3's goals:

- WP3.1: Collecting, refining and validating existing outcome measures to enable solid assessment of the value of a treatment:
 - weighting outcome measures and understanding their impact on the quality of life and care of patient segments;
 - weighting outcome measures and understanding their appropriateness for the cost of care analyses;

- development of a digital decision-making tool, based on outcomes that could be used by HTA bodies to aid in the assessment of new therapies and treatments.
- WP3.2: Analysing and validating clinical, patient-reported and real-world data to enable the development of a novel segmentation of patients to attribute to them the right level of care:
 - deployment of computational biology approaches for assessment and analysis of large multivariate datasets (e.g. outcomes-data derived from both EHRs and clinical trials) to divide patients into more precise and personalised segments;
 - development and validation of new recommendations of treatment, care and approaches for the newly-defined patient segments based on the comparative assessment of the performance of established treatments for type 1 and type 2 diabetes.
- WP3.3: Development of a clinical digital decision-making tool, based on outcomes and healthcare experience, for healthcare providers to aid in the assessment of treatment choice.

Work Package 4 – Inflammatory bowel disease

- to establish and validate a key set of key outcomes and healthcare experience measures that matter to patients in IBD, especially related to the assessment of disease severity based on patient-reported outcomes;
- to develop digital tools to collect these data directly from patients;
- to assess the acceptance and usability of these tools in patients suffering from IBD;
- to collect a set of patient-generated data using these tools and assess how these outcomes data sets compare to and complement other measures of patient outcome derived from clinical assessments, registries and EHR data;
- to better understand patient endotypes in IBD;
- to better understand how outcomes vary with patient endotypes and clinical practice and assess their potential use for improving patient care and system efficiency in the care of IBD;
- to utilise the PRO data to develop a simple scoring algorithm to indicate a patient's risk of not showing an adequate response to their existing IBD therapy (and which could prompt his/her treating physician to re-evaluate the treatment strategy);
- to support the development of digital decision-making tools which can be used by healthcare professionals in clinical practice for more personalised treatment based on patient and disease characteristics, treatment history and risk factors.

Work Package 5 – Side effects of chemotherapy and immuno-oncology

- to establish and validate a key set of core, patient-relevant, outcomes and health care experience measures that matter to patients with chemotherapy and immune-oncology side effects, and to develop digital tools to collect these data directly from patients;
- to assess the acceptance and usability of these tools in patients suffering from the side effects of chemotherapy or immune-oncology;
- to collect a set of patient-generated data using these tools and assess how these outcome data sets compare to and complement other measures of patient outcomes derived from clinical assessments, registries and EHR data;
- to better understand how outcomes and experience with healthcare vary across patients and across clinical practice and assess the potential for improving patient care and system efficiency in the care of cancer patients;
- to better understand patient segments across chemotherapy or immuno-oncology side effects;

- to support the development of digital decision-making tools which can be used by healthcare professionals in clinical practice for more personalised treatment of patients with side effects of chemotherapy or immune-oncology.

Work Package 6 – Observatory management: communication and analysis

- establish the operation of the observatories, including continuous support to patients and other stakeholders for using the technology, collecting feedback and data;
- generate regular publications to demonstrate the value added of the observatories and the lessons learned;
- manage the gateway for users of the data (including patient-level data, whether identifiable or anonymised, and aggregated data) to be able to access the data;
- define the appropriate operational and maintenance plan to ensure the technical, organisational and financial sustainability after completion of the project. Explore with partners possible expansion into additional diseases as well as possible integration with EHR and registry data.

Work Package 7 – Project management

Take responsibility for overall project management of the project, including (but not limited to) finance management for the project as a whole; meeting management and organisation (for the project as a whole); administration of communication activities; and supporting the reporting to and communication with the IMI office. WP7 will not be responsible for managing the activities of the individual work packages.